
The CuRe Trial: Cellular Therapy for In Utero Myelomeningocele Repair

Grant Award Details

The CuRe Trial: Cellular Therapy for In Utero Myelomeningocele Repair

Grant Type: Clinical Trial Stage Projects

Grant Number: CLIN2-12129

Project Objective: The objective of this project is to treat six patients in the Phase 1 portion of a Phase 1/2 clinical trial testing the use of placental mesenchymal stem cell-seeded dural matrix to surgically repair the defect in myelomeningocele patients in utero. The patients will be monitored for 30 months postnatally, assessing both safety and efficacy outcomes.

Investigator:

Name:	Diana Farmer
Institution:	University of California, Davis
Type:	PI

Disease Focus: Neurological Disorders, Spina Bifida

Human Stem Cell Use: Adult Stem Cell

Award Value: \$8,996,474

Status: Pre-Active

Grant Application Details

Application Title: The CuRe Trial: Cellular Therapy for In Utero Myelomeningocele Repair

Public Abstract:**Therapeutic Candidate or Device**

Allogeneic Placenta-derived Mesenchymal Stem Cells Seeded on Cook Biodesign® Dural Graft Extracellular Matrix (PMSC-ECM)

Indication

Myelomeningocele (MMC) -or Spina Bifida -diagnosed prenatally

Therapeutic Mechanism

Placenta-derived mesenchymal stem cells (PMSCs) act by a paracrine mechanism, secreting a variety of growth factors, cytokines, and extracellular vesicles. This secretory profile is unique to PMSCs and is responsible for protecting motor neurons from apoptosis, which occurs due to chemical and mechanical trauma when motor neurons are exposed to the intrauterine environment. PMSC treatment increases the density of motor neurons in the spinal cord, leading to improved motor function.

Unmet Medical Need

The current standard of care in utero surgery, while promising, still leaves 58% of patients unable to walk independently. There is an extraordinary need for a therapy that prevents or lessens the severity of the devastating and costly lifelong disabilities associated with the disease.

Project Objective

Phase 1 trial completed

Major Proposed Activities

- Enrollment of 6 patients to demonstrate safety and preliminary efficacy of PMSC-ECM product
- FDA-compliant manufacturing and testing studies of the PMSC-ECM product

Statement of Benefit to California:

There is a high incidence of MMC in CA with 39.1% of the population being of Hispanic or Latino descent, a demographic that is affected by MMC at a disproportionately high rate. The cost to CA is approximately \$532,000 per child, but for many, the cost may be several million dollars due to ongoing treatment. Indirect costs include pain and suffering, specialized childcare, and lost time of unpaid caregivers. A therapy for MMC would relieve the tremendous emotional and economic cost burden to CA.

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